HEALEY ALS Platform Trial

Weekly Q&A – June 16, 2022
Guest Speaker

Bruce Rosenblum
EAP Patient Advisory Committee Member
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial.

- **Screening**
  - Regimen A (n=160 for each regimen)
  - Regimen B
  - Regimen C
  - Regimen D
  - Regimen E

- **Regimen Assignment**
  - 3:1 Randomization within each Regimen

  - **Regimen**
    - Regimen A
    - Regimen B
    - Regimen C
    - Regimen D
    - Regimen E

- **Zilucoplan**
  - Placebo

- **Verdiperstat**
  - Placebo

- **CNM-Au8**
  - Placebo

- **Pridopidine**
  - Placebo

- **Trehalose**
  - Placebo

- **Shared Placebo**
Enrollment Updates (as of June 16, 2022)

• 77 individuals have signed informed consent

• 48 individuals have been randomized within Regimen E

Thank You

This breakthrough trial would not be possible without your participation

Your partnership in research is what keeps us filled with passion, dedication, and the commitment to uncover new promising treatments for ALS

Every research participant, whether on the active drug or placebo, plays a critical role in making the hope of finding a cure for ALS a reality
37 Sites Currently Activated for Regimen E

(as of 6/16/22)

Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!

- Lehigh Valley Health Network
- Mass General Hospital
- University of Kansas
- University of Maryland
- California Pacific Medical Center
- Northwestern University
- Virginia Commonwealth University
- University of Nebraska
- Washington University
- Wake Forest University
- Hospital for Special Care
- Saint Alphonsus Regional
- University of Massachusetts
- Duke University
- Barrow Neurological Institute
- Georgetown University
- Texas Neurology
- Beth Israel Deaconess Medical Center
- SUNY Upstate
- Spectrum Health
- Henry Ford Hospital
- Essentia Health
- University of Southern California
- University of South Florida
- University of Colorado
- Providence Brain and Spine
- University of Minnesota
- Loma Linda University
- University of Iowa
- Swedish Medical Center
- Ohio State University
- University of Cincinnati
- Thomas Jefferson University
- UC San Francisco
- Mayo Rochester
- University of Washington
- Vanderbilt University
Checking Site Status Online

List of Participating Sites

Sites marked "Recruiting" are currently enrolling participants in regimen E.

Sites marked "Active, Not recruiting" are active in the platform (for example, they are following participants in regimens A-D) but are not enrolling new participants at this time. Most active sites are expected to start enrolling in regimen E soon.

<table>
<thead>
<tr>
<th>Site</th>
<th>State</th>
<th>Enrollment Status</th>
<th>Contact Information</th>
</tr>
</thead>
<tbody>
<tr>
<td>Lehigh Valley Health Network</td>
<td>PA</td>
<td>Recruiting</td>
<td>Andrew Orzel (610-402-9543)</td>
</tr>
<tr>
<td>Penn State Hershey</td>
<td>PA</td>
<td>Active, Not recruiting</td>
<td>Heidi Runk</td>
</tr>
</tbody>
</table>
Patient Navigation
Central resource for people living with ALS

Phone: 833-425-8257 (HALT ALS)
E-mail: healeyalsplatform@mgh.harvard.edu

Weekly webinar registration: https://bit.ly/3r6Nd2L
ALS Link sign-up: https://bit.ly/3o2Ds3m

Upcoming Guest Speakers:
June 23rd- Jackie Whitesell, MD (Investigator at Saint Alphonsus Medical Center, Idaho)
June 30th- Gale Kittle, RN, MPH (Director, Outcomes Measurement Training)
July 14th- Eufrosina Young, MD (Investigator at SUNY Upstate, New York)
The ALS Association/Northeast ALS Consortium Educational Webinar

**Healey ALS Platform Trial Regimen E: A Participant's Guide**

**Friday, June 17, 2022**
5:00 - 6:00 PM EDT

The purpose of this webinar is to provide an overview of how SLS-005 (trehalose) may help to slow ALS and illustrate what potential participants can expect when enrolling in Regimen E of the Healey ALS Platform Trial. The presenters will review some of the unique aspects of the regimen related to the intravenous administration.

Register Below:

https://bit.ly/3aeT5kc

Recording will later be available under “educational webinars” on neals.org